WS3.5 Patients diagnosed with CF in adulthood; clinical course following diagnosis and treatment at a regional UK centre

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Introduction: Adult diagnosed patients represent a unique population that differ in their clinical, genetic and prognostic characteristics compared to those diagnosed in childhood. With a different spectrum of CFTR mutations, infections and organ involvement response to standard CF treatments is unknown.
Aims: To determine clinical status at diagnosis and assess response to CF centre treatment over a 4 yr period.
Methods: All patients diagnosed after 16 yrs of age (2005 to 2011) were included. Clinical data at diagnosis were collected from electronic patient records (EMIS®). Best FEV_1, weight and BMI for each subsequent 12 month interval recorded.
Results: 20 patients (12 male, 75% PS, median (range) age at diagnosis 30(17–60) yrs, BMI 21.8(16.7–31.9), FEV_1 66(43–101) and FVC 87(62–106)% predicted) were included. 10 patients were diagnosed due to bronchiectasis and 10 non-respiratory [infertility (6), family history (3) and malabsorption (1)]. Median BMI at diagnosis was greater in those with PS vs. PI (22.0 vs. 19.2, $p < 0.01$). BMI increased from 21.8 at diagnosis to 24.6 at yr 4. Median weight gain over 4 yrs was 6.25 kg (5 kg PS vs. 12.3 kg PI, $p < 0.005$). FEV_1 was greater at diagnosis in those with a non-respiratory diagnosis (93 vs. 60% predicted, $p < 0.005$). In patients with bronchiectasis FEV_1 increased from 60 to 75% predicted at 1 yr; this increase was maintained at 4 yrs (78% predicted). No increase in FEV_1 in those with a non-respiratory diagnosis.
Conclusions: Adult diagnosed CF patients do demonstrate significant improvements in both nutritional and respiratory status in response to CF centre treatment and care, especially those with PI and bronchiectasis.

WS3.6 The CF-ABLE score: a novel clinical prediction tool in cystic fibrosis

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Determining prognosis and predicting outcomes in cystic fibrosis (CF) is a complex issue and very few clinically applicable models have been created. Our aim was to identify clinical variables that predict outcome and create a simple prediction rule. Single-centre consecutive CF patients ($n = 49$) were studied over the period 2004–2010. Baseline clinical parameters were gathered and Forced Expiratory Volume in 1 second (FEV1) measurements were analysed longitudinally. The FEV1 decline showed a "tipping-point" with a cut-off of 52.8%. Poor outcome was defined as death or transplantation. Using FEV1, Body Mass Index (BMI), Age and Number of Exacerbations in last 3 months (NoE) the ABLE score was created. The score was validated in 370 patients from the National CF registry (area under the ROC curve=82.1% $p < 0.001$). A practical validation by two blinded independent CF clinicians was also performed on randomised sub-sample of the patients.
The ABLE score employs clinical parameters that are measured at every clinic visit. It is scored from 0 to 7 points. If FEV1 is below 52.8% then 3.5 points are added, 1.5 points is added if NoE is more than 1 in 3 months, if BMI is less than 20 or Age less than 24 then each gets 1 point. There is a linear increased risk of death or referral to transplant as patients score higher ($r \approx 0.80$, $p < 0.003$).
Patients with a low score have almost no chance of death or needing lung transplantation within 4 years, however as the score increases the risk almost doubles per point added. Patients in the validation cohort who scored over 5 had a 26.3% risk of poor outcome within 4 years. This simple score predicts outcome in CF patients better than FEV1 alone.

WS3.7 Survival at low lung function in cystic fibrosis: cohort study from 1995 to 2010

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Background: Outcomes in cystic fibrosis have seen an improvement over the past 20 years but there remains uncertainty on the outcomes of those with severe disease. An FEV_1 of less than 30% predicted has been generally accepted as the cut-off for consideration for lung transplantation based on a 2 year survival of 50%. Recently questions have been raised on the significance of this threshold.
Aim: To evaluate the survival of patients with CF whose lung function has deteriorated to an FEV_1 below 30% predicted and to explore factors associated with any change in survival.
Method: Cohort study of CF subjects in the South of England whose FEV_1 was first observed to be less than 30% predicted between 1 January 1995 and 31 December 2009. Survival was examined through to 31 December 2010 in four-year sub-cohorts. At point of entry data was collated on characteristics and current therapies.
Results: 202 participants in total (111 (55%) male) with median follow up of 5.13 years. Median survival has not been reached for the latest cohort (2004–2009) but 5 year survival for the first cohort (1995–1999) was 37% compared to 64% for the latest cohort (2004–2009). Use of recombinant DNase was significantly associated with a reduced risk of death (hazard ratio 0.64 95% CI 0.41 to 0.99). Significantly increased risk was associated with BMI <19 (HR 2.19, 1.35 to 3.53) and long term home oxygen therapy (HR 1.90, 1.15 to 3.94).
Conclusions: The use of FEV_1 <30% predicted as the most important indicator for the timing of lung transplantation is outdated and other factors should be considered. Survival continues to improve, with the importance of airway clearance treatments and nutrition reinforced.

WS3.8 The effect of socioeconomic deprivation on clinical outcomes in cystic fibrosis

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Background: Deprivation has an adverse effect on many chronic diseases. Social class adversely affects life expectancy in CF. We hypothesised that deprivation would adversely affect clinical outcomes in CF.
Methods: We assessed all 194 children and adolescents with CF under our care in South and Mid Wales. Height, weight and body mass index standard deviation scores (SDS) were generated; both FEV_1% predicted and slope of change in FEV_1% over 3 years were calculated. We assessed 3 measures of socioeconomic deprivation: the Welsh Index of Multiple Deprivation: Child Index (WIMD) a robust measure which calculates deprivation within seven domains; Townsend score and Carstairs index. Relationships between continuous variables were investigated through correlation and multiple linear regression. WIMD upper and lower quartiles were generated and compared using un-paired t-test.
Results: Patients with CF lived in significantly more deprived locations than normal – mean WIMD rank 801 v 948 (95% CI diff −225, −68; $p < 0.001$). However there was no significant relationship between clinical variables and any of the 3 indices of deprivation. Patients living in the WIMD lowest deprivation quartile were significantly taller than those in the highest quartile (mean ht SDS −0.84 v −1.50, 95% CI diff −1.26, −0.06, $p = 0.03$), but there were no other significant differences.
Conclusions: Our CF population live in significantly more deprived communities than the normal population, but there was little evidence of a deleterious effect of deprivation on clinically relevant CF outcomes. We speculate that the close monitoring of CF patients might ameliorate the effects of deprivation.